Ex vivo transduced autologous human CD34+ hematopoietic stem cells for treatment of cystinosis

Grant Award Details

Ex vivo transduced autologous human CD34+ hematopoietic stem cells for treatment of cystinosis

Grant Type: Late Stage Preclinical Projects

Grant Number: CLIN1-09230

Project Objective: To complete pre-clinical study of cysteamine in the mouse model, manufacture gene-modified HSCs for autologous transplantation in cystinosis patients, and develop and submit an IND to the FDA.

Investigator:

Name:	Stephanie Cherqui
Institution:	University of California, San Diego
Type:	PI

Disease Focus: Cystinosis, Genetic Disorder, Metabolic Disorders, Pediatrics, Toxicity

Human Stem Cell Use: Adult Stem Cell

Award Value: $5,273,189

Status: Active

Progress Reports

Reporting Period: OM #01

View Report

Grant Application Details

Application Title: Ex vivo transduced autologous human CD34+ hematopoietic stem cells for treatment of cystinosis

Public Abstract:

Therapeutic Candidate or Device

Transduced Hematopoietic Stem Cells from Peripheral Blood Stem Cells of adults and pediatric patients with cystinosis

Indication

Autologous hematopoietic stem cell gene therapy for patients with cystinosis

Therapeutic Mechanism

Direct transfer of proteins from interstitial macrophages to host cells via long tubular protrusions called tunneling nanotubes, transplantion of autologous HSC modified to express functioning cystinosin as a safer approach to provide long-term protection to the kidney, eye, thyroid and other tissues.

Unmet Medical Need

Standard of Care is cysteamine therapy which has severe side effects. Patients still require kidney transplants, develop hypothyroidism, diabetes, and neuromuscular disorders. Gene-modified HSCs would provide a safe and effective one-time life-long therapy for children and adults with cystinosis.

Project Objective

Complete the pre-clinical studies to support IND.

Major Proposed Activities

- Support and implement a pre-clinical study of cysteamine in the mouse model.
- Develop the CMC for supporting the gene-modified HSCs for autologous transplantation clinical trial for patients with cystinosis.
- Prepare and Submit Documents for Regulatory Approvals

Statement of Benefit to California:

Though patients with cystinosis in California and the United States are rare, the technology to undergo gene-modified HSCs for autologous transplantation is cutting edge research and utilizes the California resources albeit scientist and laboratories of UCSD, UCLA, and other CRO organizations in California. Once this technology is studied in the cystinosis population, the technology can be used in other applications of lysosomal disorders.

Source URL: https://www.cirm.ca.gov/our-progress/awards/ex-vivo-transduced-autologous-human-cd34-hematopoietic-stem-cells-treatment